New noncoding RNA chemical entity for heart failure with preserved ejection fraction.

Grant Award Details

New noncoding RNA chemical entity for heart failure with preserved ejection fraction.

Grant Type: Quest - Discovery Stage Research Projects

Grant Number: DISC2-13009

Investigator:

Name:	Eduardo Marban
Institution:	Cedars-Sinai Medical Center
Type:	PI

Disease Focus: Heart Disease, Heart failure

Human Stem Cell Use: Adult Stem Cell

Award Value: $1,397,412

Status: Pre-Active

Grant Application Details

Application Title: New noncoding RNA chemical entity for heart failure with preserved ejection fraction.

Public Abstract:

Research Objective

Modified synthetic noncoding RNA molecule

Impact

Heart failure with preserved ejection fraction

Major Proposed Activities

- Lead optimization
- Perform extensive preclinical testing and select a therapeutic candidate.
- Develop and test preliminary potency assays based on mechanistic insights.
- Demonstration of injury-modifying bioactivity in a clinically-relevant human progenitor cell population.
- Optimize formulation and dosing for intravenous delivery, assessing biodistribution.
- Optimize formulation and dosing for oral delivery. Plus Activity 7: Regulatory planning.

Statement of Benefit to California:	The target indication is heart failure with preserved ejection fraction (HFpEF), a highly lethal disease refractory to medical intervention. HFpEF is increasing in prevalence, and now accounts for most hospital admissions for heart failure in California. HFpEF disproportionately afflicts disadvantaged populations (women, Blacks and Latinos, and the elderly). Because the therapeutic candidate is universally applicable, the societal benefits of success here are expected to be profound.

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